Apr 3rd, 2:47 PM - 2:59 PM

Improving Access to Rare Disease Clinical Trials Through Telemedicine

Dylan Haynes
Oregon Health & Science University

Christina Topham
Oregon Health & Science University

Teri Greiling
Oregon Health & Science University

Title: Improving Access to Rare Disease Clinical Trials Through Telemedicine

Authors: Haynes D; Topham C; Greiling T

Affiliation(s): Oregon Health & Science University, Department of Dermatology

Keywords: Rare disease; randomized clinical trial; access; telemedicine

Abstract

Introduction: Rare diseases affect nearly 400 million individuals worldwide. The geographic density of any single disease, however, can prohibit access to care and research efforts. Telemedicine has improved access barriers in the clinical setting, though its application in rare disease trials has not been evaluated.

Objective: To assess the impact on access to care of videoconferencing in a rare disease clinical trial.

Materials and Methods: Preliminary analysis was performed on the first five participants to complete our ongoing trial (NCT03485976) evaluating the efficacy of ixekizumab for the treatment of pityriasis rubra pilaris. Participants were required to travel to Oregon Health & Science University (OHSU) for their enrollment and 24-week endpoint visits. All remaining visits were completed by remote videoconferencing. Questionnaires were administered to patients and providers following study visits and graded on true/false or 5-point Likert scales.

Results: Mean flight distance between participant hometown and OHSU was 1787 miles (range 207-2436). All participants (5/5) reported having the necessary equipment to complete videoconferencing visits prior to learning about the study. All participants (5/5) were strongly satisfied with the quality, safety, and privacy of their remote visits at the 24-week exit survey. Providers reported being unable to perform an accurate full-body skin exam during 4/15 visits, though did not report any instances of compromised patient safety (0/15). One participant reported they would not have enrolled had all visits required travel to OHSU.

Conclusions: Inclusion of remote videoconferencing in clinical trials may improve recruitment and access to care for individuals with rare disease.

Analysis (ignore)

tele_q_prov_4 Provider satisfied (strong dis to strong agr)

```
. tabulate tele_q_prov_4
```

Overall, I was satisfied with the remote visit	Freq.	Percent	Cum.
Strongly Disagree	1	6.67	6.67
Disagree	4	26.67	33.33
Neutral	1	6.67	40.00
Agree	7	46.67	86.67
Strongly Agree	2	13.33	100.00
Total	15	100.00	

tele_q_prov_5 Preferred performing in person (strong dis to strong agr)

I would have preferred performing this visit in person	Freq.	Percent	Cum.
Neutral	8	53.33	53.33
Agree	7	46.67	100.00
Total	15	100.00	

tele_q_prov_6 Able to accurately assess (strong dis to strong agr)

I was able to accurately assess the patient over the videoconferencing software	Freq.	Percent	Cum.
Disagree	4	26.67	26.67
Neutral	7	46.67	73.33
Agree	4	26.67	100.00
Total	15	100.00	

tele_q_prov_7 Able to express concern (strong dis to strong agr) 14 strong agr, 1 agr

tele_q_prov_8 Felt safety was compromised (strong dis to strong agr) 14 strong dis, 1 dis

tele_questions_obs_1 Would enroll even if all travel to OHSU (y/n) y,n,y,y,y

tele_questions_obs_6 Used video for healthcare before (y/n) all no

tele_questions_obs_10 patient satisfied (strong dis to strong agr) all strong agree

tele_questions_obs_11 Able to express concern (strong dis to strong agr) all strong agree

tele_questions_obs_12 Knew who to contact if emergency (strong to strong) all strong agree

tele_questions_obs_13 Felt comfortable inj (strong to strong) all strong agree

tele_questions_obs_15 comf using new tech (strong to strong) agree, neut, agree, strong ag, strong ag

upload_pt

vid_quality very bad, poor, fair, good, excellent

accuracy t/f

Variable	Obs	Mean	Std. Dev.	Min	Max
tele_q_pro~4	15	3.333333	1.234427	1	5
tele_q_pro~5	15	3.466667	.5163978	3	4
tele_q_pro~6	15	3	.7559289	2	4
tele_q_pro~7	15	4.933333	.2581989	4	5
tele_q_pro~8	15	1.066667	.2581989	1	2
tele_ques~_1	5	.8	.4472136	0	1
tele_ques~_2	5	1	0	1	1
tele_ques~_6	5	0	0	0	0
tele_ques~_7	5	1	0	1	1
tele_quest~9	0				
tele_ques~10	5	5	0	5	5
tele_ques~11	5	5	0	5	5
tele_ques~12	5	5	0	5	5
tele_ques~13	5	5	0	5	5
tele_ques~15	5	4.2	.83666	3	5